

Heart disease, blindness, Alzheimer's and muscular dystrophy research get multi-million dollar funding boost from Stem Cell Agency

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San Francisco, CA –Researchers working on using stem cells to treat heart disease, muscular dystrophy, Alzheimer's and retinitis pigmentosa have been awarded \$63 million in funding by California's stem cell agency, the California Institute for Regenerative Medicine (CIRM) at a meeting of its governing board on September 5th and 6th.

One of the projects, using stem cells to try and reduce scarring and regenerate heart muscle after a heart attack, has already been approved by the Food and Drug Administration to move into clinical trials.

"There are more than 6 million people in the US living with heart disease so this approach has the potential to make an enormous impact on public health," says Jonathan Thomas, PhD, JD, Chair of the Independent Citizens Oversight Committee (ICOC), CIRM's governing body. "Our goal is to fund and support the best science, and the best scientists to produce therapies for a wide range of diseases. What they learn, what they discover, will ultimately benefit all of us."

The heart disease project was previously funded by CIRM as a Disease Team project using the patient's own cells. The researchers have since changed their methodology, using donor cells to develop the therapy. With that in mind the ICOC voted to fund this for phase II clinical trials following NIH funding of the phase I safety approach. The retinitis pigmentosa research project has moved rapidly forward from a CIRM translational project to one nearly ready for a phase I/II clinical study.

"These are excellent examples of CIRM moving projects efficiently and effectively through the translational pipeline and into clinical trials," says Alan Trounson, PhD, President of CIRM. "We expect many other projects to evolve to first-in-human therapeutics that hopefully will deliver significant benefit to patients"

The muscular dystrophy application was awarded funding as an Early Translation project, which focuses on proof of concept of a new therapy.

The ICOC also awarded more than \$38 million to 28 other projects as part of its Basic Biology awards program, focusing on basic research, to gain a better understanding of the different kinds of stem cells and how to work with them.

"This kind of basic research is essential to helping us answer some essential questions about stem cells," says Pat Olson, PhD, Executive Director of Scientific Studies at CIRM. "The knowledge we gain from these studies will ultimately inform other work and advance our understanding of the fundamental mechanism of stem cell biology, and move us ever closer to knowing how best to use stem cells to help patients."

In addition the ICOC approved \$6.7 million in funding for a Research Leadership Award. The Leadership Awards are intended to help draw talented faculty members to California institution. In this case it will bring Dennis Steindler to the Parkinson's Institute in Sunnyvale to do research that will help better understand the importance and uses of stem cells for the treatment of Parkinson's disease, and at the same time get a better insight into their role in disease repair and causation.

The meeting celebrated the tenure of two ICOC members and welcomed a new member to the board. Ted Love, MD and David Serrano Sewell, JD were thanked for their contributions to the stem cell agency, and praised for their dedication and compassion; the ICOC said the skills and expertise they brought played an important role in helping shape CIRM. The Board also welcomed its newest member, Anne-Marie Duliege, MD. She is currently the Chief Medical Officer for biotech company Affymax and brings with her considerable clinical trial expertise to the Board and hands-on experience with the FDA approval process.

Disease Team II applications funded

Disease Team II Award project summaries

Application	Researcher	Institution	Total Funds Requested
DR2A-05735	Rachel Smith	Capricor, Inc	\$19,782,136
DR2A-05739	Henry Klassen	University of California, Irvine	\$17,306,668
DR2A-05426	Stanley Nelson	University of California, Los Angeles	\$6,000,000
DR2A-05416	Alexandra Capela	StemCells, Inc.	\$20,000,000
Total			\$63,088,804

Research Leadership application funded

Research Leadership award project summaries

Application	Researcher	Institution	Total Funds Requested
LA1-06535	Dennis Steindler	The Parkinson's Institute	\$6,718,471

Basic Biology IV applications funded

Basic Biology IV award project summaries

Application	Researcher	Institution	Total Funds Requested
RB4-05763	Howard Chang	Stanford University	\$1,386,627
RB4-05779	George Sen	University of California, San Diego	\$1,080,000
RB4-05785	Deepak Lamba	Buck Institute for Age Research	\$1,526,319
RB4-05825	Roel Nusse	Stanford University	\$1,195,811
RB4-05855	Jeremy Reiter	University of California, San Francisco	\$1,387,800
RB4-05886	Gerald Crabtree	Stanford University	\$1,392,426
RB4-05901	Benoit Bruneau	The J. David Gladstone Institutes	\$1,708,560
RB4-05990	Barbara Panning	University of California, San Francisco	\$1,285,214
RB4-06016	Robert Tjian	University of California, Berkeley	\$1,220,968
RB4-06028	Miguel Ramalho-Santos	University of California, San Francisco	\$1,184,883
RB4-06035	Sheng Ding	The J. David Gladstone Institutes	\$1,708,560
RB4-06036	Michael Cleary	Stanford University	\$1,244,455
RB4-06041	Ben Barres	Stanford University	\$1,385,517
RB4-06045	Gene Yeo	University of California, San Diego	\$1,393,200

RB4-06079	Steven Finkbeiner	The J. David Gladstone Institutes	\$1,506,420
RB4-06087	Anne Brunet	Stanford University	\$1,414,044
RB4-06093	Michelle Monje	Stanford University	\$1,264,248
RB4-06102	Alexander Dunn	Stanford University	\$1,064,224
RB4-06133	Kathrin Plath	University of California, Los Angeles	\$1,382,400
RB4-06144	Maike Sander	University of California, San Diego	\$1,391,400
RB4-06209	Owen Witte	University of California, Los Angeles	\$1,382,400
RB4-06215	Eduardo Marbıçn	Cedars-Sinai Medical Center	\$1,367,604
RB4-06244	Ananda Goldrath	University of California, San Diego	\$1,161,000
RB4-06276	Huei-sheng Chen	Sanford-Burnham Medical Research Institute	\$1,582,606
RB4-06345	Miles Wilkinson	University of California, San Diego	\$1,360,450
RB4-06158	David Traver	University of California, San Diego	\$1,363,698
RB4-06277	Yanhong Shi	Beckman Research Institute of City of Hope	\$1,367,172
RB4-05764	Deborah Lieu	University of California, Davis	\$1,334,880
Total			\$38,042,886

Disease Team and Early Translational Award brief descriptions

Capricor

This company worked with the team from Cedars-Sinai to make a version of its Cardiospheres in which the cells are separated, which they call Cardiosphere Derived Cells. Using donor cells makes the therapy easier than using a patient's own cells and makes it accessible to more patients – an important factor in treating heart attack patients where speed is important. In June 2012 they received FDA permission to begin a clinical trial in patients who had a heart attack relatively recently, and who already had clear signs of reduced heart function. This CIRM award will fund a clinical trial to test the safety and effectiveness of this therapy.

More information about CIRM funding for heart disease

University of California, Irvine

For retinitis pigmentosa the team plans to use donor tissue to isolate cells that are part way down the path from neural stem cells to adult eye tissue. These retinal progenitor cells would be grown in large quantities in the lab and then injected into the eye. The team suggests the cells could help in two ways. They may be able to protect the photoreceptors not yet damaged by the disease, and they may be able to form new photoreceptors to replace those already lost. They plan to complete testing needed to apply to the FDA to begin an early phase trial of the therapy.

More information about CIRM funding for blindness

University of California, Los Angeles

The team plans a therapy that can give Duchenne Muscular Dystrophy (DMD) patients a correct version of dystrophin, a protein that plays a key role in muscle cell health, but is defective in DMD. They plan to alter genes in the muscle cells so as to result in normal production of dystrophin. The initial goal of this project was to complete preclinical studies looking at safety and effectiveness, and to file an Investigational New Drug application (IND) within four years. However, the ICOC felt these goals were too ambitious and instead recommended this be re-categorized as an Early Translational study, one more focused on proof of concept rather than drug

development.

More information about CIRM funding for muscular dystrophy

StemCells, Inc.

This team proposes to inject adult brain stem cells into the front section of the brain. They have shown in animal models that these cells produce chemicals that appear to help protect existing nerve cells. They plan to use this award to produce cells that meet the high standards needed to get FDA approval and to complete the testing required before they can begin clinical trials in people. More information about Alzheimer's disease.

More information about CIRM funding for Alzheimer's disease

About CIRM: CIRM was established in November 2004 with the passage of Proposition 71, the California Stem Cell Research and Cures Act. The statewide ballot measure, which provided \$3 billion in funding for stem cell research at California universities and research institutions, was overwhelmingly approved by voters, and called for the establishment of an entity to make grants and provide loans for stem cell research, research facilities, and other vital research opportunities. A list of grants and loans awarded to date may be seen here: <https://www.cirm.ca.gov/grants>.

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